
Cellular Reprogramming: Recent Advances in Modeling Neurological Diseases.

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Funding Grants: Developing a drug-screening system for Autism Spectrum Disorders using human neurons, Cellular tools to study brain diseases affecting synaptic transmission

Public Summary:

This paper provides a review on the field of induced pluripotent stem cells for disease modeling.

Scientific Abstract:

The remarkable advances in cellular reprogramming have made it possible to generate a renewable source of human neurons from fibroblasts obtained from skin samples of neonates and adults. As a result, we can now investigate the etiology of neurological diseases at the cellular level using neuronal populations derived from patients, which harbor the same genetic mutations thought to be relevant to the risk for pathology. Therapeutic implications include the ability to establish new humanized disease models for understanding mechanisms, conduct high-throughput screening for novel biogenic compounds to reverse or prevent the disease phenotype, identify and engineer genetic rescue of causal mutations, and develop patient-specific cellular replacement strategies. Although this field offers enormous potential for understanding and treating neurological disease, there are still many issues that must be addressed before we can fully exploit this technology. Here we summarize several recent studies presented at a symposium at the 2011 annual meeting of the Society for Neuroscience, which highlight innovative approaches to cellular reprogramming and how this revolutionary technique is being refined to model neurodevelopmental and neurodegenerative diseases, such as autism spectrum disorders, schizophrenia, familial dysautonomia, and Alzheimer's disease.

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